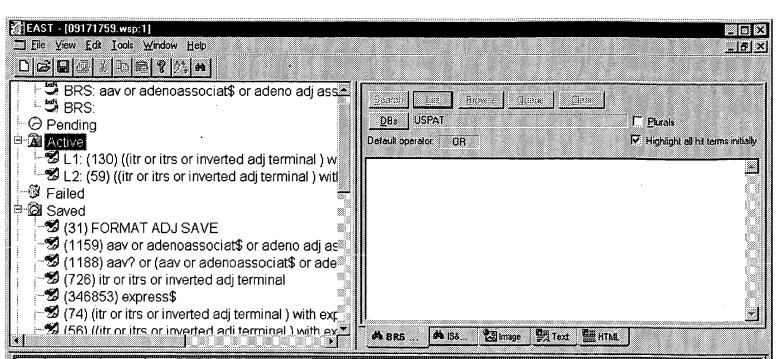
effective promoter with the CFTR gene into AAV vectors. We engineered and regions that may contain nonessential sequences. Functional analyses showed tested 20 CFTR mini-genes containing deletions that were targeted to to reduce the size of the CFTR cDNA to allow the incorporation of an epithelial cells in animal models and in patients. However, the level of CFTR gene together with a promoter. In this study, we described a strategy CFTR gene expression has been low because the vector cannot accommodate the effective in transferring the cystic fibrosis gene (CFTR) into airway 0027-8424 Journal Code: 7505876 CA 94143, USA. ? s au=dong j? S1 407 AU=DONG J? America (UNITED STATES) Aug 18 1998, 95 (17) p10158-63, ISSN Department of Laboratory Medicine, University of California, San Francisco 09949408 98374322 PMID: 9707617 ? t s2/7/2 removal, customized scheduling. See HELP ALERT. that carry shortened CFTR genes. DIALOG(R)File 155:MEDLINE(R) ? s aav and s1 \*File 155: Alert feature enhanced for multiple files, duplicates File 155:MEDLINE(R) 1966-2002/Aug W4 Adeno-associated virus (AAV)-based vectors have been shown to be Record type: Completed Main Citation Owner: NLM Languages: ENGLISH Document type: Journal Article Contract/Grant No.: DK/HL46177; DK; NIDDK; DK47766; DK; NIDDK Proceedings of the National Academy of Sciences of the United States of Gene Therapy Core Center for Cystic Fibrosis and Genetic Diseases and Zhang L; Wang D; Fischer H; Fan P D; Widdicombe J H; Kan Y W; Dong J Y Efficient expression of CFTR function with adeno-associated virus vectors \$0.41 Estimated cost this search \$0.37 Estimated cost File1 Set Items Description \$0.41 Estimated total session cost 0.105 DialUnits \$0.04 TELNET 30aug02 13:29:12 User208669 Session D2097.1 \$0.37 0.105 DialUnits File1 998 AAV 5 AAV AND SI

> of which exceed the packaging limit of an AAV vector. expression. Our strategy also may be applicable to other genes, the sizes Logoff: level 02.08.23 D 13:30:29 ? log hold vectors should circumvent the limitations of AAV vector for CFTR and more effectively transferred CFTR function into target cells. These new virions more efficiently, generated higher titers of transducing virions, expressed the CFTR gene more efficiently than larger vectors or a vector in also demonstrated that smaller AAV/CFTR vectors with a P5 promoter genes into target cells and demonstrated high levels of CFTR expression. We probability, time voltage dependence, and regulation by cAMP. By using an sequence. The CFTR mini-gene with combined deletions was packaged into AAV which CFTR gene was expressed from the AAV inverted terminal repeat AAV vector with a P5 promoter, we transduced these short forms of CFTR function and the characteristics of a wild-type CFTR, as measured by open that four of the shortened CFTRs (one with combined deletions) retained the Record Date Created: 19980917 \$2.25 Estimated total session cost 0.481 DialUnits \$1.84 Estimated cost this search \$0.43 TELNET \$1.41 Estimated cost File155 30aug02 13:30:29 User208669 Session D2097.2 \$1.20 0.376 DialUnits File155 \$0.21 6 Types **\$**0.21 1 Type(s) in Format 7 \$0.00 5 Type(s) in Format 6



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1	BRS	L1	130	((itr or itrs or inverted adj terminal) with express\$) and (aav? or (aav or adenoassociat\$ or adeno adj associat\$))	USPAT	2002/08/30 14:29	
2	BRS	L2	59	((itr or itrs or inverted adj terminal) with express\$) and (aav? or (aav or adenoassociat\$ or adeno adj associat\$))	US-PGPUB	2002/08/30 14:29	